CIRM invests $89 million in stem cell and gene therapy research

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The Board of the California Institute for Regenerative Medicine (CIRM) approved investing nearly $89 million in projects from its Clinical and Translation programs at its April meeting.

Nearly $39 million will support four projects in the agency's Clinical program, which speeds up support and provides funding for eligible stem cell and gene therapy-based projects through any stage of clinical trial activity.

Included in the awards is a $15 million grant to support Combangio, Inc. in a Phase 2 clinical trial to evaluate the safety and efficacy of a topical therapy produced from human bone marrow stem cells to treat persistent corneal epithelial defect (PCED).

PCED results in a delay of corneal healing due to trauma, disease or other factors. This delay initially results in severe pain and redness but can progress to infection, corneal ulcers, and blindness. PCED remains a significant burden on patients and caregivers and no effective therapies exist that address all causes.

One of the major contributing factors to development of PCED is diabetes. Over 3 million Californians have diabetes, and approximately 43% are at risk for associated PCED.

The proposed therapy—known as KPI-012—aims to reduce or eliminate pain, restore normal corneal wound healing, provide a curative solution for corneal lesions, and prevent vision loss.

In this trial, 90 PCED patients will be enrolled to test the safety and efficacy of KPI-012.

This month's clinical awards include two clinical-stage projects and two preclinical projects. That brings the number of CIRM-funded clinical trials to 90.

The successful applicants in CIRM's Clinical program this month are:

Application	Program Title	Principal Investigator / Institution	Amount
CLIN1-14602	Clinical Translation of Autologous Regenerative Pluripotent Stem Cell Therapy for Blindness	Schwartz, Steven - University of California, Los Angeles	$6,000,000
CLIN1-14764	Treatment of the TMJ disc complex	Athanasiou, Kyriacos - University of California, Irvine	$6,000,000
CLIN2-14315	Reduced intensity conditioning with JSP191 prior to TCRαβ+ T-cell/CD19+ B-cell depleted hematopoietic stem cell transplant for Fanconi Anemia patients	Porteus, Matthew - Stanford University	$11,813,964
CLIN2-14516	Phase 2b Clinical Study of KPI-012 Topical Ophthalmic Human Mesenchymal Stem Cell Secretome for the Treatment of Persistent Corneal Epithelial Defect	Brazzell, R. Kim - Combangio, Inc.	$15,000,000

The Board also awarded nearly $50 million to support 10 projects in its Translational program.

The goal of CIRM's Translational program is to support promising stem cell-based or gene projects that accelerate completion of translational stage activities necessary for advancement to clinical study or broad end use. Those can include therapeutic candidates, diagnostic methods or devices and novel tools that address critical bottlenecks in research.

The successful applicants in CIRM's Translational program this month are:

Application	Program Title	Principal Investigator / Institution	Amount
TRAN1-14062	Escape-Resistant Oligonucleotide Therapy (ONT) for Cytomegalovirus (CMV) Disease in Hematopoietic Stem-Cell and Solid-Organ Transplant Patients	Weinberger, Leor - VxBiosciences inc.	$3,977,180
TRAN1-14609	Enhanced Autologous Pancreatic Islet Transplantation and Survival for Diabetes Mellitus Therapy	Park, Walter – Stanford University	$6,056,713
TRAN1-14613	Novel T cell receptor-STEM T cell immunotherapy in lung cancer	Wang, Rongfu - University of Southern California	$5,689,540
TRAN1-14623	Telomerase mRNA for short telomere related pulmonary fibrosis	Ramunas, John - Rejuvenation Technologies, Inc.	$3,984,942
TRAN1-14625	Hematopoietic Stem/Progenitor Cell-Based Chimeric Antigen Receptor Gene Therapy for HIV Infection	Kitchen, Scott - University of California, Los Angeles	$6,140,723
TRAN1-14649	Extracellular Vesicle-Based Therapy for Corneal Scars	Deng, Sophie - University of California, Los Angeles	$5,779,276
TRAN1-14671	Development of Autologous Cell Replacement Therapy for Parkinson's Disease: Path to Personalized Treatment	Baetge, Emmanuel - BrainXell Therapeutics	$3,841,110
TRAN1-14698	Hematopoietic Stem Cell Gene Therapy for Wiskott Aldrich Syndrome	Riggan, Luke - ImmunoVec	$3,999,899
TRAN1-14716	Targeting multiple myeloma with BCMA-CAR NK cells expressing a GPRC5D-NKG2D bispecific antibody	Caligiuri, Michael - Beckman Research Institute of City of Hope	$6,036,001

TRAN1-14710	AAV Gene Therapy for Treating Congenital Hereditary Endothelial Dystrophy (CHED) associated with Biallelic SLC4A11 Mutations	Aldave, Anthony - University of California, Los Angeles	$4,338,166

About the California Institute for Regenerative Medicine (CIRM)

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov
